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## February 2014 Newsletter

### CIRM February Newsletter

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### CIRM Funds More Than \$66 Million in Genomics Initiative and Basic Research

At its January 29 meeting, the CIRM governing Board awarded \$40 million to create a new Center of Excellence in Stem Cell Genomics by a team of experts from seven different major California institutions. The Center of Excellence will focus on bridging the fields of genomics – studying the complete genetic make-up of a cell or organism – with stem cell research. The goal is to use these tools to gain a deeper understanding of the disease processes in cancer, diabetes, heart disease and mental health, and ultimately to try and find safer and more effective ways of using stem cells in medical research and therapy. The Center of Excellence consists of Stanford University and the Salk Institute for Biological studies as the joint Principal Investigators; U.C. San Diego, the Scripps Research Institute, the J. Craig Venter Institute and Illumina Inc., all in San Diego, will also collaborate on the project; U.C. Santa Cruz will run the Data Coordination and Management component. The Board also approved more than \$27 million in funding for 27 Basic Biology V awards. These go to researchers trying to advance the field by tackling significant, unresolved issues in human stem cell biology.

- [Read our press release and blog about the awards](#)

### Two CIRM-Funded Companies Sign Collaboration Agreements with Big Pharma to Fund Clinical Trials

2014 got off to a great start for two of our grantees. On January 6th, Capricor Therapeutics announced that it signed a \$337.5 million collaboration agreement (\$12.5 million up front) with Janssen Biotech, a division of pharmaceutical giant Johnson & Johnson. In December, Capricor got approval to move ahead with a Phase 2 clinical trial for its heart disease treatment. CIRM is funding that trial through a \$19.8 million Disease Team Award. The trial, due to start early this year, is designed to test a stem cell treatment on patients who have experienced a heart attack, to see if the treatment is both safe and reduces scar tissue on the heart.

Then on January 9th, Sangamo BioSciences announced that it reached an agreement on a global collaboration with Biogen Idec, a move that could speed up work to develop cures for both sickle cell disease and Beta-thalassemia. Biogen Idec will give Sangamo \$20 million upfront with the possibility of an additional \$300 million if the company's research hits certain milestones in development, sales and royalties. Last May Sangamo got a \$6.3 million award from CIRM to develop a method of correcting faulty genes that lead to sickle cell disease and beta-thalassemia.

- [Read our blog about the Capricor-Janssen agreement](#)
- [Read our blog about the Sangamo-Biogen Idec agreement](#)

### Stem Cell Therapies for Diabetes: CIRM Hosts Live Google Hangout



On January 23rd, CIRM hosted a live Google Hangout On Air about recent progress in stem cell based strategies for diabetes therapies. The live conversation included four guest speakers: Drs. Kevin D'Amour and Howard Foyt from ViaCyte, Dr. Francisco Prieto a physician and researcher and the Patient Advocate for Diabetes for our governing Board, and Chris Stiehl, who has been living with type 1 diabetes for 54 years. With the help of CIRM funding, Viacyte has developed a device that is implanted under the skin and secretes insulin and other hormones to keep blood glucose levels under control. The device contains cells derived from human embryonic stem cells that become insulin-producing pancreatic beta cells. Clinical trials in people are anticipated to begin this year.

- [Watch the hangout](#)
- [Read our blog about the hangout](#)

#### Board Swears in Biotech Veteran and Actress/Alzheimer's Activist





At the January CIRM governing Board meeting, Joe Panetta and Lauren Miller were sworn in as its newest members. Mr. Panetta, President and CEO of Biocom, was chosen because of his more than 30 years of experience in the biotech, government and corporate world. Mr. Panetta takes over the seat left vacant by the death of Duane Roth last year. Miller, an actress, screen writer and activist committed to raising awareness about Alzheimer's disease, takes over the Alzheimer's patient advocate seat left vacant by the departure of Leeza Gibbons. Both Panetta and Miller were appointed to the Board by Governor Jerry Brown.

- [Read our press release and blog about Mr. Panetta's appointment](#)
- [Read our press release and blog about Ms. Miller's appointment](#)

### **Breaking Bottlenecks to a Better Cell Source for Blood Stem Cell Transplantation: A CIRM Report**



In January, CIRM science officers Kelly Shepard and Soheli Talib released a report based on an August 2013 CIRM mini-symposium that focused on overcoming the scientific and technical bottlenecks that still exist with transforming embryonic and induced pluripotent stem cells into hematopoietic stem cells. This cell type is used in bone marrow transplantation to treat blood disorders and cancers such as leukemia. Breaking this bottleneck could provide an unlimited, compatible, off-the-shelf source of blood stem cells compared to the current situation in which finding a healthy source of immune-matched donor tissue can be difficult or impossible.

- [Read the CIRM report \[pdf\]](#)

### **1700 and Counting: The Latest Publications on CIRM-funded Research**

With over 1700 published discoveries, CIRM grantees are making steady progress on understanding the molecular basis of stem cell biology and moving stem cell-based therapies toward clinical trials. Here are example of CIRM-funded research that were published in January along with a press release from the grantees' home institution or local media and a CIRM Stem Cell Research Blog:

**January 3rd - *Cell Stem Cell***

- Union Tribune San Diego: Embryonic stem cell rejection problem fixed, study says
- CIRM Blog: Putting the brakes on immune rejection may help accelerate embryonic stem cell-based therapies

#### **January 7th - *Proceedings of the National Academies of Science (PNAS)***

- UCSD Release: Biomaterials Get Stem Cells to Commit to a Bony Future
- CIRM Blog: Understanding how stem cells become new bone could make the process more efficient, cheaper

#### **January 14th - *Nature Cell Biology***

- CIRM Blog: To be or not to be a stem cell: Sanford-Burnham lab finds new mechanism

#### **Upcoming CIRM Events**

**March 13th:** The CIRM governing board will hold its second of seven meetings of 2014. This meeting will take place in Burlingame, CA. The agenda and details will be posted to the **meetings page** 10 days before the event.

#### **CIRM Funding Opportunities**

**RFA 13-07: CIRM Alpha Stem Cell Clinics Network: Coordinating and Information Management Center** - Applications due March 14th, 2014 at 5:00pm (Pacific). Applications will only be accepted from applicants who 1) submitted an Letter of Intent (LOI) in December and 2) were notified by CIRM that they are eligible to apply. CIRM conducted a webinar for prospective applicants on Nov. 5, 2013 to address questions about both RFA 13-06 and 13-07 and the application process. A recording of the webinar, presentation slides, and the Q&A transcript are now available on the RFA 13-07 page.

**RFA 13-06: CIRM Alpha Stem Cell Clinics Network: Alpha Stem Cell Clinics** - Applications due March 14th, 2014 at 5:00pm (Pacific). Applications will only be accepted from applicants who 1) submitted an Letter of Intent (LOI) in December and 2) were notified by CIRM that they are eligible to apply. CIRM conducted a webinar for prospective applicants on Nov. 5, 2013 to address questions about both RFA 13-06 and 13-07 and the application process. A recording of the webinar, presentation slides, and the Q&A transcript are now available on the RFA 13-06 page.

**RFA 13-04: External Innovation Pilot Program** - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section VIII.A. of the RFA for evaluation criteria. **This RFA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.**

**RFA 13-03: CIRM Strategic Partnership III Awards** - CIRM intends to offer repeat calls under this initiative every 6-9 months. The focus, scope and objective may differ with each solicitation. **CIRM conducted a webinar for prospective applicants on August 7, 2013** to address questions about the RFA and application process. Click here to watch webinar and view Q & A.

**PA 12-08: CIRM Patent Assistance Fund Awards** - Application opportunities on a monthly basis until June 30, 2014  
**Eligibility: Only Technology Transfer Officials at current CIRM Grantee institutions are eligible to apply for funds under this program.**

**PA 08-06: CIRM Conference Grants** - Applications for conference grants will be accepted, reviewed and approved periodically  
 Get more info about CIRM RFAs  
 Sign up to receive CIRM funding announcements

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